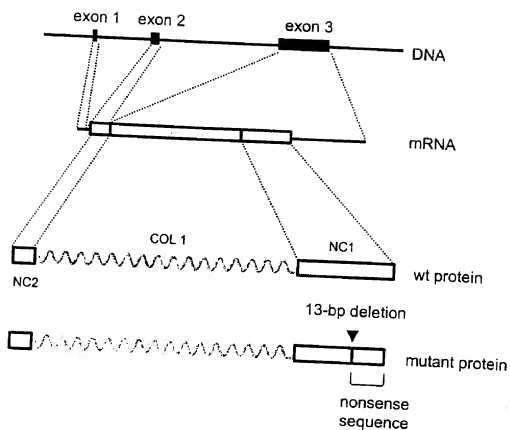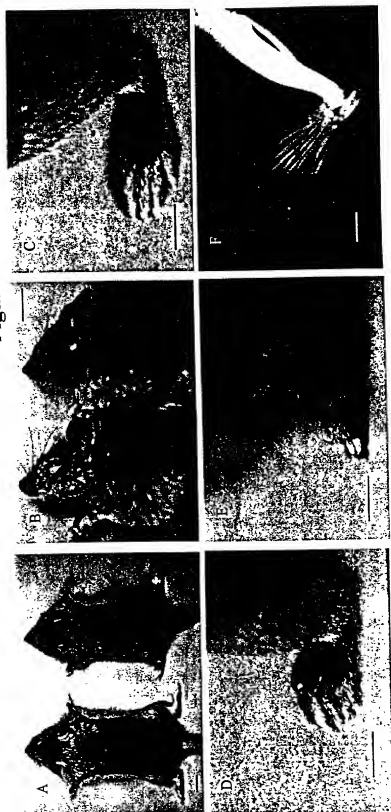**Figure 1**

Figure 2

```
---
GTC ATG CCT GAT GGC TTC ATA AAG GCA GGC CAG AGG CCC AGG CTT TCT GGG ATG CCG CTT      mouse wt
val met pro asp gly phe ile lys ala gly gln arg pro arg leu ser gly met pro leu

540
GTC AGT GCT AAC CAC GGG GTA ACA GGT ATG CCC GTG TCT GCT TTT ACT GTC ATT CTC TCT      mouse wt
val ser ala asn his gly val thr gly met pro val ser ala phe thr val ile leu ser

560
AAA GCT TAC CCA GCA GTA GGT GCC CCC ATC CCA TTT GAT GAG ATT CTG TAC AAT AGG CAG      mouse wt
lys ala tyr pro ala val gly ala pro ile pro phe asp glu ile leu tyr asn arg gln

580
CAG CAT TAC GAC CCA AGA TCT GGT ATC TTT ACC TGT AAG ATC CCA GGC ATA TAC TAT TTC      mouse wt
gln his tyr asp pro arg ser gly ile phe thr cys lys ile pro gly ile tyr tyr phe

600
TCC TAC CAC GTG CAT GTG AAA GGG ACT CAC GTT TGG GTA GGC CTG TAT AAG AAC GGC ACG      mouse wt
ser tyr his val his val lys gly thr his val trp val gly leu tyr lys asn gly thr

                                                                          ACA         mouse 13del
                                                                          thr         human 13del
                                                                          thr

620
CCT ACG ATG TAC ACG TAT GAT GAG TAC AGC AAA GGC TAC CTG GAT CAG GCT TCA GGG AGT      mouse wt
pro thr met tyr thr tyr asp glu tyr ser lys gly tyr leu asp gln ala ser gly ser

CGT ATG ATG AGT ACA GCA AAG GCT ACC TGG ATC AGG CTT CAG GGA GTG CAA TCA TGG AGC      mouse 13del
arg met met ser thr ala lys ala thr trp ile arg leu gln gly val gln ser trp ser    human 13del
pro met met asn thr pro lys ala thr trp ile arg leu gln gly val pro ser ser ile

640
GCA ATC ATG GAG CTC ACA GAA AAT GAC CAG GTA TGG CTC CAA TTG CCC AAT GCA GAA TCA      mouse wt
ala ile met glu leu thr glu asn asp gln val trp leu gln leu pro asn ala glu ser

TCA CAG AAA ATG ACC AGG TAT GGC TCC AAT TGC CCA ATG CAG AAT CAA ACG GCC TCT ACT      mouse 13del
ser gln lys met thr arg tyr gly ser asn cys pro met gln asn gln thr ala ser thr    human 13del
ser gln lys met thr arg cys gly ser ser phe pro met pro ser gln met ala tyr thr

660
AAC GGC CTC TAC TCC TCT GAG TAC GTC CAC TCG TCC TTC TCA GGA TTC CTA GTG GCT CCC      mouse wt
asn gly leu tyr ser ser glu tyr val his ser ser phe ser gly phe leu val ala pro

                                               671
CCT CTG AGT ACG TCC ACT CGT CCT TCT CAG GAT TCC TAG                                  mouse 13del
pro leu ser thr ser thr arg pro ser gln asp ser STOP                                 human 13del
pro leu ser met ser thr pro leu ser gln asp ser STOP

680
ATG TGA                                                                              mouse wt
met STOP
```
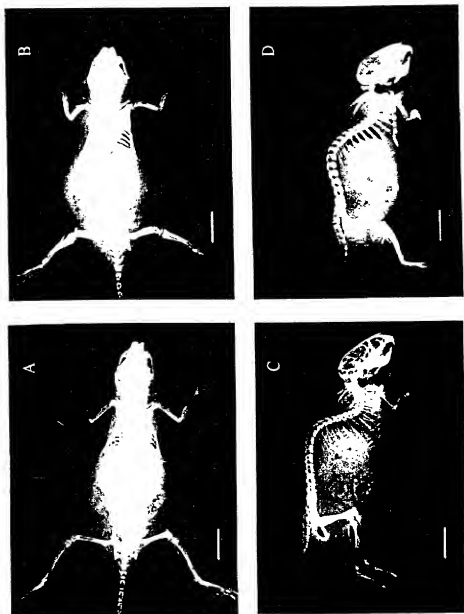
Figure 3

Figure 4

Figure 5

Figure 6

Figure 7

Figure 8

Non-transgenic   Transgenic

Fig 8a   Fig 8b

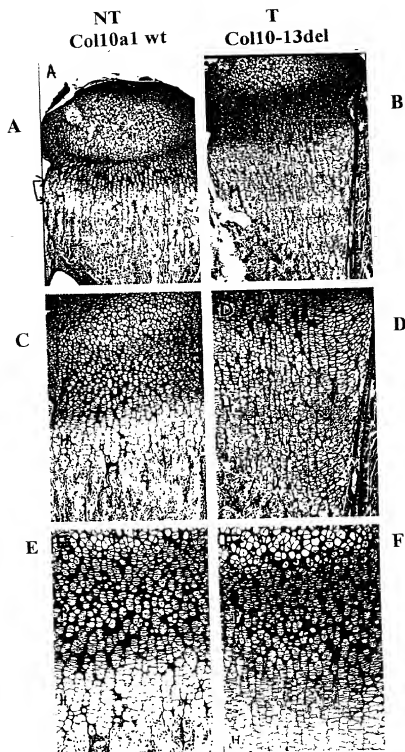Fig 8c   Fig 8d

Fig 8e   Fig 8f
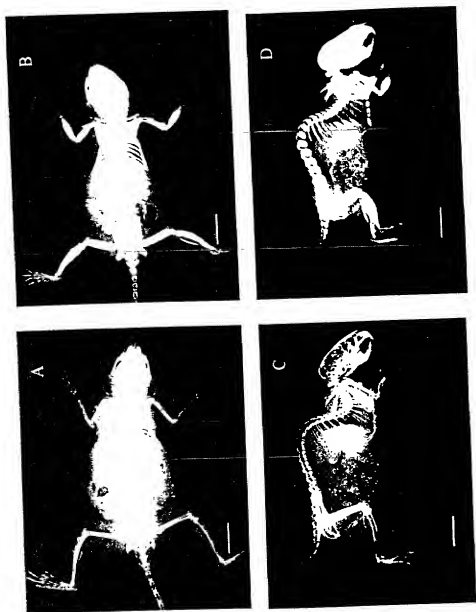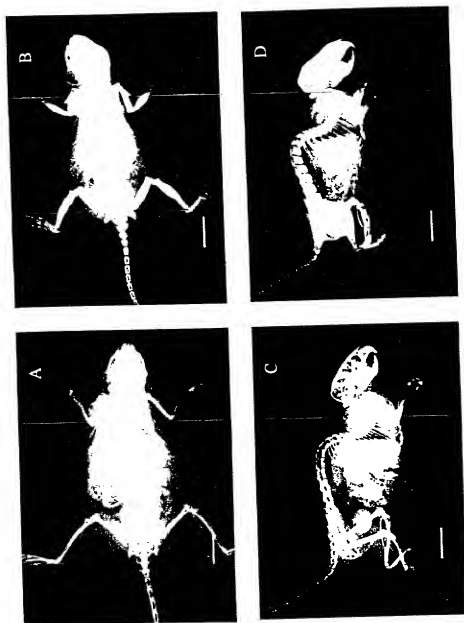
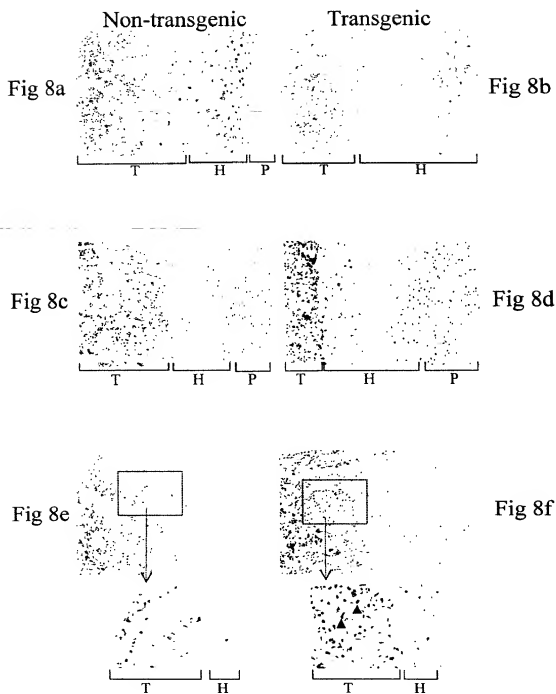## Figure 9

Fig 9a
(100X)
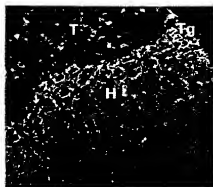
Fig 9b
(100X)

Fig 9c
(100x)

Fig 9d
(1000X)